

Treatment of Overactive Bladder (OAB)

FINAL COMPREHENSIVE RESEARCH PLAN

July 3rd, 2015

Study Team: Pharmacoeconomic Unit

Research Questions

- RQ1. What is the current evidence for the comparative cost-effectiveness of pharmacologic treatments for overactive bladder (OAB) syndrome?
- RQ2. Based on a de novo economic model, what is the comparative cost-effectiveness of pharmacologic treatments for OAB syndrome?
- RQ3. What is the budget impact of alternative policies for reimbursing pharmacotherapies for the management of OAB syndrome?
- RQ4. Based on a de novo economic model, what is the cost-effectiveness of alternative policies for reimbursing pharmacologic treatments for OAB syndrome?

Methods

Systematic Review of Published Economic Evaluations

To address RQ1 we will conduct a systematic review of the available literature on the cost-effectiveness of pharmacotherapy options for the treatment of OAB syndrome. Specifically, therapeutic options will include anticholinergic (ACH) medications (oxybutynin, tolterodine, fesoterodine, trospium, darifenacin, solifenacin, flavoxate), mirabegron, botulinum toxin type A, and combination therapy (ACH+mirabegron), as compared with each other or placebo.

A search of the medical literature will be conducted in MEDLINE (OVID interface, indexed, in-process and other non-indexed citations, 1946 onwards), EMBASE (OVID interface, 1947 onwards), NHS EED, and Tufts CEA registry in order to capture all relevant literature based on the NHS EED recommended search strategy. This literature search will be carried out by coupling a standard search strategy for identifying economic studies with the clinical search terms adopted by the clinical review. Moreover, a search of grey literature sources such as the CADTH and NICE websites, as well as hand-searching of reference lists of retrieved studies will supplement the electronic database search.

Two independent reviewers will screen the titles and abstracts of citations retrieved by the initial literature search, and potentially relevant full-text articles will be obtained and screened for inclusion in the economic appraisal by the same two reviewers. Any disagreements will be resolved by discussion or the involvement of a third reviewer.

Extracted studies will then be further reviewed with studies excluded for lack of context or for not being full economic evaluations.

Critical appraisal of economic evidence will entail identifying common methodological issues within included studies. Each study will be assessed through a three step process: initial assessment for validity, assessment of study quality, and assessment of study's pertinence to the decision question. Comparators will include anticholinergic (ACH) medications (oxybutynin, tolterodine, fesoterodine, trospium, darifenacin, solifenacin, flavoxate), mirabegron, botulinum toxin type A, and combination therapy (ACH+mirabegron), as compared with each other or placebo or other non pharmacological interventions.

Emphasis will be placed on the strength and quality of evidence addressing the cost-effectiveness of anticholinergic (ACH) medications (oxybutynin, tolterodine, fesoterodine, trospium, darifenacin, solifenacin, flavoxate), mirabegron, botulinum toxin type A, and combination therapy (ACH+mirabegron), as compared with each other or placebo, for the management of OAB syndrome.

De novo Economic Evaluation

We will develop a de novo economic model to assess the cost-effectiveness of alternative pharmacotherapies for the treatment of OAB syndrome

The economic model will build on previous analyses. We will construct a Markov model which will model disease progression. Natural history data relating to disease progression will be combined with treatment effectiveness data from the clinical review conducted as part of this class review. Disease progression will be modelled through 61 health states which will be hybrid states relating to the presence and daily number of incontinence episodes and the daily number of micturitions as well as death as an absorbing state. Data on the effect of treatments on the daily number of micturitions, incontinence episodes and side effects will be required from the companion network meta analysis. Utility values will be sourced from the literature. We will adopt a time horizon of 1 year for the base case with 5 years in sensitivity analysis. All OAB treatments including combinations for which clinical data from the companion systematic review are available will be included.

Costs and utilities associated with disease progression will be derived from the literature. Analysis will be conducted from the perspective of the Ministry of Health with sensitivity analysis presented from a quasi societal perspective incorporating the cost of incontinence pads. Results will be presented as incremental cost per quality adjusted life years gained. Detailed deterministic sensitivity analysis will be conducted along with Monte Carlo simulation methods to determine decision uncertainty.

Reimbursement-based Budget Impact Analysis

The aim of this portion of the pharmacoeconomic review is to develop a budget impact analysis that will facilitate the reimbursement decision-making process. Emphasis will be placed on identifying the budget impact of alternative approaches to the current reimbursement status of pharmacologic treatments for patients with OAB syndrome. This will be achieved through a three stage process.

1. Forecasting of current expenditure for pharmacologic treatments for OAB syndrome.
 - We will obtain data on current usage of pharmacotherapy options for treating OAB syndrome from OPDP to allow identification of the number of claims, number of claimants, total costs, and drug unit costs in a given year (broken down quarterly).
2. Identification of candidate reimbursement strategies
 - The second stage will involve identifying alternative approaches to reimbursement of combination therapies. This will rely heavily on strategies identified during the scoping assessment along with further consultation with OPDP. Reimbursement strategies could be general (applied to all products) or specific (targeted at specific products), and consideration may be given to the availability of generics and changes to listing.
3. Assessment of budget impact of candidate scenarios
 - Using the techniques adopted in step 1, we will forecast the budget expenditure on pharmacotherapies for the treatment of OAB syndrome for each alternative reimbursement strategy.

Reimbursement-based Economic Evaluation

The aim of this component is to utilize data from the de novo economic model to allow identification of the optimal reimbursement criteria through considering cost-effectiveness as criteria with a focus on reimbursement strategies not just interventions. Analysis will identify

the cost-effectiveness of alternative approaches to the current reimbursement status of pharmacotherapies for treating patients with OAB syndrome.

Deliverables

We will provide a written report detailing methods adopted, results, discussion and summary policy recommendations. The report will comprise a two-page executive summary followed by a detailed technical report.

Timelines

Our work will commence on acceptance of this proposal. The review of economic evidence will be completed within 6 weeks of project onset. The de novo economic model will be developed and populated within 12 weeks of commencement, and the initial forecasting of drug expenditures will be completed within the same time frame. Both of these components are scheduled to coincide with the completion of the clinical review. Moreover, reimbursement based economic modelling will be completed between 12 and 16 weeks to allow delivery of an aligned final report at 16 weeks. Reanalyses and revisions of the final report will be available 4 weeks after receipt of stakeholder reviews.